Pre-symptomatic Infants with Spinal Muscular Atrophy Achieved Same Motor Milestones as Healthy Children After Treatment with Evrysdi™ in RAINBOWFISH

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- JEWELFISH results demonstrated stabilization in motor function in broad SMA population -
- Evrysdi has proven efficacy in adults, children and babies two months and older -
- Results presented at CureSMA Conference -

SOUTH PLAINFIELD, N.J., June 11, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced positive interim results from the ongoing RAINBOWFISH trial that demonstrated pre-symptomatic infants with spinal muscular atrophy (SMA) treated with Evrysdi were able to reach motor milestones such as sitting without support, rolling, crawling, standing unaided and walking independently.

"It is incredible to see that pre-symptomatic SMA infants are able to achieve developmental milestones consistent with healthy children," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics. "These results broaden our knowledge of Evrysdi in babies younger than two months of age and adds to the proven efficacy data in a broad range of SMA patients."

Additionally, results presented from a 12-month interim analysis of the JEWELFISH safety study demonstrated overall stabilization in motor function and rapid and sustained increases in SMN protein. These patients began treatment with Evrysdi following previous treatments, including Spinraza® or Zolgensma® and are representative of the real-world, broad and heterogeneous SMA population with a high degree of motor impairment at baseline.

Evrysdi is designed to treat SMA by increasing and sustaining the production of the SMN protein, which is found throughout the body and is critical for maintaining healthy motor neurons and movement. Evrysdi has been approved for the treatment of patients with SMA, aged 2 months and older by the FDA and the EMA. Roche leads the clinical development of Evrysdi as part of a collaboration with the SMA Foundation and PTC Therapeutics. Evrysdi is marketed in the United States by Genentech, a member of the Roche Group.

About Spinal Muscular Atrophy (SMA)
Spinal muscular atrophy (SMA) is a severe, progressive neuromuscular disease that can be fatal. It affects approximately 1 in 10,000 babies and when untreated is the leading genetic cause of infant mortality. SMA is caused by a mutation of the survival motor neuron 1 (SMN1) gene, which leads to a deficiency of SMN protein. This protein is found throughout the body and is essential to the function of nerves that control muscles and movement. Without it, nerve cells cannot function correctly, leading to progressive muscle weakness over time. Depending on the type of SMA, an individual's physical strength and their ability to walk, eat or breathe can be significantly diminished or lost.

About Evrysdi™ (risdiplam)
Evrysdi™ is a survival motor neuron 2 (SMN2)-directed RNA splicing modifier designed to treat SMA caused by mutations in chromosome 5q that lead to SMN protein deficiency. Evrysdi™ is designed to distribute evenly to all parts of the body, including the central nervous system (CNS). Evrysdi™ is administered daily at home in liquid form by mouth or feeding tube. The U.S. Food and Drug Administration approved Evrysdi™ (risdiplam) in August 2020 for the treatment of spinal muscular atrophy for adults and children 2 months and older. Evrysdi™ (risdiplam) is marketed in the United States by Genentech, a member of the Roche Group.

About the Evrysdi™ (risdiplam) Clinical Studies

RAINBOWFISH (NCT03779334) is an open-label, single-arm, multi-center study, investigating the efficacy, safety, pharmacokinetics, and pharmacodynamics of risdiplam in babies (~n=25), from birth to 6 weeks old (at first dose), with genetically diagnosed SMA, who are not yet presenting symptoms. The study is currently recruiting.

JEWELFISH (NCT03032172) is an open-label exploratory trial designed to assess the safety, tolerability, pharmacokinetics (PK) and pharmacodynamics (PD) in people with SMA aged 6 months to 60 years who received other investigational or approved SMA therapies for at least 90 days prior to receiving Evrysdi™. The study has completed recruitment (n=174).

In addition to JEWELFISH and RAINBOWFISH, Evrysdi™ has been evaluated in a broad range of people with SMA, including in:

FIREFISH (NCT02913482) is an open-label, two-part pivotal clinical trial in infants with Type 1 SMA. Part 1 was a dose-escalation

study in 21 infants with the primary objective of assessing the safety profile of risdiplam in infants and determining the dose for Part 2. Part 2 is a pivotal, single-arm study of risdiplam in 41 infants with Type 1 SMA treated for two years followed by an open-label extension. The primary objective of Part 2 was to assess efficacy as measured by the proportion of infants sitting without support after 12 months of treatment, as assessed in the Gross Motor Scale of the Bayley Scales of Infant and Toddler Development - Third Edition (BSID-III) (defined as sitting without support for five seconds). The study met its primary endpoint.

SUNFISH (NCT02908685) is a two-part, double-blind, placebo controlled pivotal study in people aged 2 to 25 years with Types 2 or 3 SMA. Part 1 (n=51) determined the dose for the confirmatory Part 2. Part 2 (n=180) evaluated motor function using the Motor Function Measure 32 (MFM-32) scale at 12 months. MFM-32 is a validated scale used to evaluate fine and gross motor function in people with neurological disorders, including SMA. The study met its primary endpoint.

Clinical Trial Safety Data
The safety profile of Evrysdi™ was established across FIREFISH and SUNFISH pivotal trials. The most common adverse reactions in later-onset SMA (incidence of at least 10 percent of patients treated with Evrysdi™ and more frequently than control) were fever, diarrhea, and rash. The most common adverse reactions in infantile-onset SMA were similar to those observed in later-onset SMA patients. Additionally, the most common adverse reactions (incidence of at least 10 percent) were upper respiratory tract infection, pneumonia, constipation, and vomiting.

About PTC
PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. The Company's strategy is to leverage its strong scientific expertise and global commercial infrastructure to maximize value for its patients and other stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, on Twitter at @PTCBio, and on LinkedIn.

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Forward-Looking Statements:
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this release, other than statements of historic fact, are forward-looking statements, including statements regarding: advancement of PTC's joint collaboration program in SMA, including the commercialization of any products therein or royalty or milestone payments; the future expectations, plans and prospects for PTC, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses, licensing or commercialization of its products and products candidates and other matters; PTC's strategy, future operations, future financial position, future revenues, projected costs; and the objectives of management. Other forward-looking statements may be identified by the words, "guidance", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: the outcome of pricing, coverage and reimbursement negotiations with third party payors for PTC's products or product candidates that PTC commercializes or may commercialize in the future; the enrollment, conduct, and results of ongoing studies under the SMA collaboration and events during, or as a result of, the studies that could delay or prevent further development under the program, including any regulatory submissions and commercialization with respect to Evrysdi; significant business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of PTC's products and product candidates; PTC's scientific approach and general development progress; and the factors discussed in the "Risk Factors" section of PTC's most recent Annual Report on Form 10-K, as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC. You are urged to carefully consider all such factors.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and

commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Evrysdi.

The forward-looking statements contained herein represent PTC's views only as of the date of this press release and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this press release except as required by law.

View original content:http://www.prnewswire.com/news-releases/pre-symptomatic-infants-with-spinal-muscular-atrophy-achieved-same-motor-milestones-as-healthy-children-after-treatment-with-evrysdi-in-rainbowfish-301310547.html

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